

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(10) International Publication Number
WO 2022/013443 A1

(43) International Publication Date
20 January 2022 (20.01.2022)

(51) International Patent Classification:

C07D 233/66 (2006.01) A61K 47/24 (2006.01)
C07D 213/40 (2006.01) A61K 47/28 (2006.01)
A61K 9/127 (2006.01) C12N 15/88 (2006.01)

TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,
KM, ML, MR, NE, SN, TD, TG).

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))
- in black and white; the international application as filed contained color or greyscale and is available for download from PATENTSCOPE

(21) International Application Number:

PCT/EP2021/070026

(22) International Filing Date:

16 July 2021 (16.07.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

20305825.0 17 July 2020 (17.07.2020) EP

(71) Applicant: SANOFI PASTEUR [FR/FR]; 14 Espace Hen-
ry Vallée, 69007 LYON (FR).

(72) Inventors: HAENSLER, Jean; c/o Sanofi, 54, rue La
Boetie, 75008 Paris (FR). EVEN, Luc; c/o Sanofi, 54, rue
La Boetie, 75008 Paris (FR). FRISCH, Benoît; c/o Sanofi,
54, rue La Boetie, 75008 PARIS (FR). RIPOLL, Manon;
c/o Sanofi, 54, rue La Boetie, 75008 PARIS (FR).

(74) Agent: CABINET NONY; 11 rue Saint-Georges, 75009
PARIS (FR).

(81) Designated States (unless otherwise indicated, for every
kind of national protection available): AE, AG, AL, AM,
AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ,
CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO,
DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN,
HR, HU, ID, IL, IN, IR, IS, IT, JO, JP, KE, KG, KH, KN,
KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD,
ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO,
NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW,
SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN,
TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, GH,
GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, ST, SZ, TZ,
UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ,
TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK,
EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV,
MC, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM,

(54) Title: LIPIDIC COMPOUNDS COMPRISING AT LEAST ONE TERMINAL RADICAL OF FORMULA -NH-CX-A OR -NH-
CX-NH-A, COMPOSITIONS CONTAINING THEM AND USES THEREOF

(57) Abstract: The disclosure relates to novel lipidic compounds, lipid nanoparticles (LNPs) containing thereof, and the use of the lipidic compounds or the LNPs for the delivery of nucleic acid. The lipidic compounds as disclosed herein comprise at least one terminal radical of formula (I): *-NH-CX-(NH)_n-A (I) wherein: - * - represents a single bond linking said radical of formula (I), directly or not, to to one C₁₀ to C₅₅ lipophilic or hydrophobic tail-group; - n is 0 or 1; - X is an oxygen or sulfur atom, and - A represents an optionally substituted 5- or 6-membered unsaturated heterocyclic radical or 5- or 6-membered heteroaromatic ring radical, both containing at least one nitrogen atom; or one of the pharmaceutically acceptable salts of said radical of formula (I); and with said compound that is in all the possible racemic, enantiomeric and diastereoisomeric isomer forms.



WO 2022/013443 A1